

Opening Statement of the Honorable Fred Upton
Subcommittee on Health
Hearing on “21st Century Cures: Incorporating the Patient Perspective”
July 11, 2014

(As Prepared for Delivery)

The entire purpose of our 21st Century Cures initiative is to accelerate the discovery, development, and delivery of safe and effective treatments to America's patients. We are here today to better understand how we can incorporate the most important perspective - that of patients and their families - into the conversation.

Patients should and need to play a key part of this process if we are to be successful. As one of our witnesses, Dr. Beall has noted, “Congress should work to ensure patients have a seat at the table, because no one understands a disease better than the people who suffer and fight every day.”

I would like to issue a special welcome to Pat Furlong who has continued to fight after losing her two boys, Christopher and Patrick, to Duchene. We are very humbled that you are here to help with the cures initiative. I'd also like to welcome to Dr. Marshall Summer – a parent of a child with Down's Syndrome. Parents are tireless advocates of their children and we are pleased that you are here today. Thank you and all of our witnesses for being here today.

I also would like to thank Dr. Woodcock for testifying today. Unfortunately, prior obligations prevented her from coming to Wednesday's hearing so today she will provide her expertise on both modernizing clinical trials and incorporating the perspective of patients.

As I'm sure Dr. Woodcock will explain, FDA has taken steps to incorporate the perspective of patients in the drug development process. FDA's work with Parents Project Muscular Dystrophy is a good example of collaboration, and we look forward to hearing about next steps on the guidance they put together.

However, much work remains. We would like to learn how we can leverage the successful examples of agency-patient collaboration and what other steps we can take to ensure the patient's perspective on the benefit-risk framework is thoroughly considered and incorporated throughout the cycle of the drug review process.

At our first 21st Century Cures roundtable, we learned that there are treatments for only 500 of the more than 7,000 known diseases affecting our nation's patients. Our work will not be done until we can close this gap in cures. I look forward to hearing how we can incorporate the voice of patients in this process.

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